

ABSTRACT OF THE DISCLOSURE

A method for treatment or prophylaxis of disease

~~caused by deficiency, in a subject, of an enzyme belonging to~~

the heme biosynthetic pathway, the method comprising administering, to the subject, an effective amount of a catalyst which is said enzyme or an enzymatically equivalent part or analogue thereof. The disease is selected from the group consisting of, acute intermittent porphyria (AIP), ALA deficiency porphyria (ADP), Porphyria cutanea tarda (PCT), Hereditary coproporphyria (HCP), Harderoporphyria (HDP), Variegata porphyria (VP), Congenital erythropoietic porphyria (CEP), Erythropoietic protoporphyria (EPP), and Hepatoerythropoietic porphyria (HEP). The catalyst is an enzyme selected from the group consisting of porphobilinogen deaminase (PBGD) ALA dehydratase, Uroporphyrinogen decarboxylase, Coproporphyrinogen oxidase, Coproporphyrinogen oxidase, Protoporphyrinogen oxidase, Uroporphyrinogen III synthase, Ferrochelatase and Uroporphyrinogen decarboxylase, or an enzymatically equivalent part or analogue thereof. In addition the invention relates to the use of PBGD and to a method of gene therapy.